



SEQUENCE LISTING

- (1) GENERAL INFORMATION:
 - (i) APPLICANT: Trustees of the University of Pennsylvania Wilson, James M. Fisher, Krishna J.
 - (ii) TITLE OF INVENTION: Method for Recombinant Adeno-Associated Virus-Directed Gene Therapy
 - (iii) NUMBER OF SEQUENCES: 4
 - (iv) CORRESPONDENCE ADDRESS:
 - (A) ADDRESSEE: Howson and Howson
 - (B) STREET: Spring House Corporate Cntr, PO Box 457
 - (C) CITY: Spring House
 - (D) STATE: Pennsylvania
 - (E) COUNTRY: USA
 - (F) ZIP: 19477
 - (v) COMPUTER READABLE FORM:
 - (A) MEDIUM TYPE: Floppy disk
 - (B) COMPUTER: IBM PC compatible
 - (C) OPERATING SYSTEM: PC-DOS/MS-DOS
 - (D) SOFTWARE: PatentIn Release #1.0, Version #1.30
 - (vi) CURRENT APPLICATION DATA:
 - (A) APPLICATION NUMBER: WO
 - (B) FILING DATE:
 - (C) CLASSIFICATION:
 - (vii) PRIOR APPLICATION DATA:
 - (A) APPLICATION NUMBER: US 08/708,188
 - (B) FILING DATE: 06-SEP-1996
 - (vii) PRIOR APPLICATION DATA:
 - (A) APPLICATION NUMBER: US 08/729,061
 - (B) FILING DATE: 10-OCT-1996
 - (viii) ATTORNEY/AGENT INFORMATION:
 - (A) NAME: Kodroff, Cathy A.
 - (B) REGISTRATION NUMBER: 33,980
 - (C) REFERENCE/DOCKET NUMBER: GNVPN.019CIP2PCT
 - (ix) TELECOMMUNICATION INFORMATION:
 - (A) TELEPHONE: 215-540-9200
 - (B) TELEFAX: 215-540-5818
- (2) INFORMATION FOR SEQ ID NO:1:
 - (i) SEQUENCE CHARACTERISTICS:
 - (A) LENGTH: 10398 base pairs

 - (B) TYPE: nucleic acid (C) STRANDEDNESS: double
 - (D) TOPOLOGY: unknown
 - (ii) MOLECULE TYPE: CDNA

(xi) SEQUENCE DESCRIPTION: SEQ ID NO:1:	
GAATTCGCTA GCATCATCAA TAATATACCT TATTTTGGAT TGAAGCCAAT ATGATAATGA	60
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TGACGTTTTT GGTGTGCGCC GGTGTACACA GGAAGTGACA ATTTTCGCGC GGTTTTAGGC	240
GGATGTTGTA GTAAATTTGG GCGTAACCGA GTAAGATTTG GCCATTTTCG CGGGAAAACT	300
GAATAAGAGG AAGTGAAATC TGAATAATTT TGTGTTACTC ATAGCGCGTA ATATTTGTCT	360
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ACTCCATCAC TAGGGGTTCC TTGTAGTTAA TGATTAACCC GCCATGCTAC TTATCTACAA	540
TTCGAGCTTG CATGCCTGCA GGTCGTTACA TAACTTACGG TAAATGGCCC GCCTGGCTGA	600
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GTACATCAAG TGTATCATAT GCCAAGTACG CCCCCTATTG ACGTCAATGA CGGTAAATGG	780
CCCGCCTGGC ATTATGCCCA GTACATGACC TTATGGGACT TTCCTACTTG GCAGTACATC	840
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GACCGATCCA GCCTCCGGAC TCTAGAGGAT CCGGTACTCG AGGAACTGAA AAACCAGAAA	1200
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AAGCGGTGCC GGAAAGCTGG CTGGAGTGCG ATCTTCCTGA GGCCGATACT GTCGTCGTCC	1740
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TTACGGTCAM TCCGCCGTII GIIGGGTCGG MOTHER COMMENT	





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	TGCCGTCTGA	ATTTGACCTG	AGCGCATTTT	TACGCGCCGG	AGAAAACCGC	CTCGCGGTGA	2040
	TGGTGCTGCG	TTGGAGTGAC	GGCAGTTATC	TGGAAGATCA	GGATATGTGG	CGGATGAGCG	2100
	GCATTTTCCG	TGACGTCTCG	TTGCTGCATA	AACCGACTAC	ACAAATCAGC	GATTTCCATG	2160
	TTGCCACTCG	CTTTAATGAT	GATTTCAGCC	GCGCTGTACT	GGAGGCTGAA	GTTCAGATGT	2220
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	ATCGCGTCAC	ACTACGTCTG	AACGTCGAAA	ACCCGAAACT	GTGGAGCGCC	GAAATCCCGA	2400
	ATCTCTATCG	TGCGGTGGTT	GAACTGCACA	CCGCCGACGG	CACGCTGATT	GAAGCAGAAG .	2460
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	AGCCGTTGCT	GATTCGAGGC	GTTAACCGTC	ACGAGCATCA	TCCTCTGCAT	GGTCAGGTCA	2580
•	TGGATGAGCA	GACGATGGTG	CAGGATATCC	TGCTGATGAA	GCAGAACAAC	TTTAACGCCG	2640
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	TGTATGTGGT	GGATGAAGCC	AATATTGAAA	CCCACGGCAT	GGTGCCAATG	AATCGTCTGA	2760
	CCGATGATCC	GCGCTGGCTA	CCGGCGATGA	GCGAACGCGT	AACGCGAATG	GTGCAGCGCG	2820
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	CTGGAGAGAC	GCGCCCGCTG	ATCCTTTGCG	AATACGCCCA	CGCGATGGGT	AACAGTCTTG	3120
	GCGGTTTCGC	TAAATACTGG	CAGGCGTTTC	GTCAGTATCC	CCGTTTACÁG	GGCGGCTTCG	3180
						CCGTGGTCGG	3240
	CTTACGGCGG	TGATTTTGGC	GATACGCCGA	ACGATCGCCA	GTTCTGTATG	AACGGTCTGG	3300
	TCTTTGCCGA	CCGCACGCCG	CATCCAGCGC	TGACGGAAGC	AAAACACCAG	CAGCAGTTTT	3360
	TCCAGTTCCG	TTTATCCGGG	CAAACCATCG	AAGTGACCAG	CGAATACCTG	TTCCGTCATA	3420
	GCGATAACGA	GCTCCTGCAC	TGGATGGTGG	CGCTGGATGG	TAAGCCGCTG	GCAAGCGGTG	3480
	AAGTGCCTCT	GGATGTCGCT	CCACAAGGTA	AACAGTTGAT	TGAACTGCCT	GAACTACCGC	3540
	AGCCGGAGAG	CGCCGGGCAA	CTCTGGCTCA	CAGTACGCGT	AGTGCAACCG	AACGCGACCG	3600
	CATGGTCAGA	AGCCGGGCAC	ATCAGCGCCT	GGCAGCAGTG	GCGTCTGGCG	GAAAACCTCA	3660
	GTGTGACGCT	CCCCCCCCC	TCCCACGCCA	TCCCGCATCT	GACCACCAGC	: GAAATGGATT	3720
	TTTGCATCGA	GCTGGGTAAT	AAGCGTTGGC	AATTTAACCG	CCAGTCAGGC	TTTCTTTCAC	3780





AGATGTGGAT TGGCGATAAA AAACAACTGC TGACGCCGCT GCGCGATCAG TTCACCCGTG 3840 CACCGCTGGA TAACGACATT GGCGTAAGTG AAGCGACCCG CATTGACCCT AACGCCTGGG 3900 TCGAACGCTG GAAGGCGGCG GGCCATTACC AGGCCGAAGC AGCGTTGTTG CAGTGCACGG 3960 CAGATACACT TGCTGATGCG GTGCTGATTA CGACCGCTCA CGCGTGGCAG CATCAGGGGA 4020 ARACCTTATT TATCAGCCGG ARAACCTACC GGATTGATGG TAGTGGTCAA ATGGCGATTA 4080 CCGTTGATGT TGAAGTGGCG AGCGATACAC CGCATCCGGC GCGGATTGGC CTGAACTGCC 4140 AGCTGGCGCA GGTAGCAGAG CGGGTAAACT GGCTCGGATT AGGGCCGCAA GAAAACTATC 4200 CCGACCGCCT TACTGCCGCC TGTTTTGACC GCTGGGATCT GCCATTGTCA GACATGTATA 4260 CCCCGTACGT CTTCCCGAGC GAAAACGGTC TGCGCTGCGG GACGCGCGAA TTGAATTATG 4320 GCCCACACCA GTGGCGCGGC GACTTCCAGT TCAACATCAG CCGCTACAGT CAACAGCAAC 4380 TGATGGAAAC CAGCCATCGC CATCTGCTGC ACGCGGAAGA AGGCACATGG CTGAATATCG 4440 . ACGGTTTCCA TATGGGGATT GGTGGCGACG ACTCCTGGAG CCCGTCAGTA TCGGCGGAAT 4500 TACAGCTGAG CGCCGGTCGC TACCATTACC AGTTGGTCTG GTGTCAAAAA TAATAATAAC 4560 CGGGCAGGCC ATGTCTGCCC GTATTTCGCG TAAGGAAATC CATTATGTAC TATTTAAAAA 4620 ACACAAACTT TTGGATGTTC GGTTTATTCT TTTTCTTTTA CTTTTTATC ATGGGAGCCT 4680 ACTTCCCGTT TTTCCCGATT TGGCTACATG ACATCAACCA TATCAGCAAA AGTGATACGG 4740 GTATTATTT TGCCGCTATT TCTCTGTTCT CGCTATTATT CCAACCGCTG TTTGGTCTGC 4800 TTTCTGACAA ACTCGGCCTC GACTCTAGGC GGCCGCGGG ATCCAGACAT GATAAGATAC 4860 ATTGATGAGT TTGGACAAAC CACAACTAGA ATGCAGTGAA AAAAATGCTT TATTTGTGAA 4920 ATTTGTGATG CTATTGCTTT ATTTGTAACC ATTATAAGCT GCAATAAACA AGTTAACAAC 4980 AACAATTGCA TTCATTTTAT GTTTCAGGTT CAGGGGGAGG TGTGGGAGGT TTTTTCGGAT 5040 CCTCTAGAGT CGAGTAGATA AGTAGCATGG CGGGTTAATC ATTAACTACA AGGAACCCCT 5100 AGTGATGGAG TTGGCCACTC CCTCTCTGCG CGCTCGCTCG CTCACTGAGG CCGGGCGACC 5160 ARAGGTCGCC CGACGCCCGG GCTTTGCCCG GGCGGCCTCA GTGAGCGAGC GAGCGCGCAG 5220 CAGATCTGGA AGGTGCTGAG GTACGATGAG ACCCGCACCA GGTGCAGACC CTGCGAGTGT 5280 GGCGGTAAAC ATATTAGGAA CCAGCCTGTG ATGCTGGATG TGACCGAGGA GCTGAGGCCC 5340 GATCACTTGG TGCTGGCCTG CACCCGCGCT GAGTTTGGCT CTAGCGATGA AGATACAGAT 5400 TGAGGTACTG AAATGTGTGG GCGTGGCTTA AGGGTGGGAA AGAATATATA AGGTGGGGGT 5460 CTTATGTAGT TTTGTATCTG TTTTGCAGCA GCCGCCGCCG CCATGAGCAC CAACTCGTTT 5520 GATGGAAGCA TTGTGAGCTC ATATTTGACA ACGCGCATGC CCCCATGGGC CGGGGTGCGT 5580 CAGAATGTGA TGGGCTCCAG CATTGATGGT CGCCCCGTCC TGCCCGCAAA CTCTACTACC 5640 TTGACCTACG AGACCGTGTC TGGAACGCCG TTGGAGACTG CAGCCTCCGC CGCCGCTTCA





GCCGCTGCAG CCACCGCCCG CGGGATTGTG ACTGACTTTG CTTTCCTGAG CCCGCTTGCA 5760 AGCAGTGCAG CTTCCCGTTC ATCCGCCCGC GATGACAAGT TGACGGCTCT TTTGGCACAA 5820 TTGGATTCTT TGACCCGGGA ACTTAATGTC GTTTCTCAGC AGCTGTTGGA TCTGCGCCAG 5880 CAGGTTTCTG CCCTGAAGGC TTCCTCCCCT CCCAATGCGG TTTAAAACAT AAATAAAAAA 5940 CCAGACTCTG TTTGGATTTG GATCAAGCAA GTGTCTTGCT GTCTTTATTT AGGGGTTTTG 6000 CGCGCGCGGT AGGCCCGGGA CCAGCGGTCT CGGTCGTTGA GGGTCCTGTG TATTTTTTCC 6060 AGGACGTGGT AAAGGTGACT CTGGATGTTC AGATACATGG GCATAAGCCC GTCTCTGGGG 6120 TGGAGGTAGC ACCACTGCAG AGCTTCATGC TGCGGGGTGG TGTTGTAGAT GATCCAGTCG 6180 TAGCAGGAGC GCTGGGCGTG GTGCCTAAAA ATGTCTTTCA GTAGCAAGCT GATTGCCAGG 6240 GGCAGGCCCT TGGTGTAAGT GTTTACAAAG CGGTTAAGCT GGGATGGGTG CATACGTGGG 6300 GATATGAGAT GCATCTTGGA CTGTATTTTT AGGTTGGCTA TGTTCCCAGC CATATCCCTC 6360 CGGGGATTCA TGTTGTGCAG AACCACCAGC ACAGTGTATC CGGTGCACTT GGGAAATTTG 6420 6480 TCATGTAGCT TAGAAGGAAA TGCGTGGAAG AACTTGGAGA CGCCCTTGTG ACCTCCAAGA TTTTCCATGC ATTCGTCCAT AATGATGGCA ATGGGCCCAC GGGCGGCGGC CTGGGCGAAG 6540 ATATTTCTGG GATCACTAAC GTCATAGTTG TGTTCCAGGA TGAGATCGTC ATAGGCCATT 6600 TTTACAAAGC GCGGGCGGAG GGTGCCAGAC TGCGGTATAA TGGTTCCATC CGGCCCAGGG 6660 GCGTAGTTAC CCTCACAGAT TTGCATTTCC CACGCTTTGA GTTCAGATGG GGGGATCATG 6720 TCTACCTGCG GGGCGATGAA GAAAACGGTT TCCGGGGTAG GGGAGATCAG CTGGGAAGAA 6780 AGCAGGTTCC TGAGCAGCTG CGACTTACCG CAGCCGGTGG GCCCGTAAAT CACACCTATT 6840 6900 ACCGGGTGCA ACTGGTAGTT AAGAGAGCTG CAGCTGCCGT CATCCCTGAG CAGGGGGGCC ACTTCGTTAA GCATGTCCCT GACTCGCATG TTTTCCCTGA CCAAATCCGC CAGAAGGCGC 6960 TCGCCGCCCA GCGATAGCAG TTCTTGCAAG GAAGCAAAGT TTTTCAACGG TTTGAGACCG 7020 7080 TCCGCCGTAG GCATGCTTTT GAGCGTTTGA CCAAGCAGTT CCAGGCGGTC CCACAGCTCG GTCACCTGCT CTACGGCATC TCGATCCAGC ATATCTCCTC GTTTCGCGGG TTGGGGCGGC 7140 TTTCGCTGTA CGGCAGTAGT CGGTGCTCGT CCAGACGGGC CAGGGTCATG TCTTTCCACG 7200 GGCGCAGGGT CCTCGTCAGC GTAGTCTGGG TCACGGTGAA GGGGTGCGCT CCGGGCTGCG 7260 CGCTGGCCAG GGTGCGCTTG AGGCTGGTCC TGCTGGTGCT GAAGCGCTGC CGGTCTTCGC 7320 CCTGCGCGTC GGCCAGGTAG CATTTGACCA TGGTGTCATA GTCCAGCCCC TCCGCGGCGT 7380 GGCCCTTGGC GCGCAGCTTG CCCTTGGAGG AGGCGCCGCA CGAGGGGCAG TGCAGACTTT 7440 TGAGGGCGTA GAGCTTGGGC GCGAGAAATA CCGATTCCGG GGAGTAGGCA TCCGCGCCGC 7500 AGGCCCCGCA GACGGTCTCG CATTCCACGA GCCAGGTGAG CTCTGGCCGT TCGGGGTCAA 7560

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GTCCACGCTC GGTGACGAAA AGGCTGTCCG TGTCCCCGTA TACAGACTTG AGAGGCCTGT 7680 CCTCGACCGA TGCCCTTGAG AGCCTTCAAC CCAGTCAGCT CCTTCCGGTG GGCGCGGGGC 7740 ATGACTATCG TCGCCGCACT TATGACTGTC TTCTTTATCA TGCAACTCGT AGGACAGGTG 7800 CCGGCAGCGC TCTGGGTCAT TTTCGGCGAG GACCGCTTTC GCTGGAGCGC GACGATGATC 7860 GGCCTGTCGC TTGCGGTATT CGGAATCTTG CACGCCCTCG CTCAAGCCTT CGTCACTGGT 7920 CCCGCCACCA AACGTTTCGG CGAGAAGCAG GCCATTATCG CCGGCATGGC GGCCGACGCG 7980 CTGGGCTACG TCTTGCTGGC GTTCGCGACG CGAGGCTGGA TGGCCTTCCC CATTATGATT 8040 CTTCTCGCTT CCGGCGCAT CGGGATGCCC GCGTTGCAGG CCATGCTGTC CAGGCAGGTA 8100 GATGACGACC ATCAGGGACA GCTTCAAGGA TCGCTCGCGG CTCTTACCAG CCTAACTTCG 8160 ATCACTGGAC CGCTGATCGT CACGGCGATT TATGCCGCCT CGGCGAGCAC ATGGAACGGG 8220 TTGGCATGGA TTGTAGGCGC CGCCCTATAC CTTGTCTGCC TCCCCGCGTT GCGTCGCGGT 8280 GCATGGAGCC GGGCCACCTC GACCTGAATG GAAGCCGGCG GCACCTCGCT AACGGATTCA 8340 CCACTCCAAG AATTGGAGCC AATCAATTCT TGCGGAGAAC TGTGAATGCG CAAACCAACC 8400 CTTGGCAGAA CATATCCATC GCGTCCGCCA TCTCCAGCAG CCGCACGCGG CGCATCTCGG 8460 GCAGCGTTGG GTCCTGGCCA CGGGTGCGCA TGATCGTGCT CCTGTCGTTG AGGACCCGGC 8520 TAGGCTGGCG GGGTTGCCTT ACTGGTTAGC AGAATGAATC ACCGATACGC GAGCGAACGT 8580 GAAGCGACTG CTGCTGCAAA ACGTCTGCGA CCTGAGCAAC AACATGAATG GTCTTCGGTT 8640 TCCGTGTTTC GTAAAGTCTG GAAACGCGGA AGTCAGCGCC CTGCACCATT ATGTTCCGGA 8700 TCTGCATCGC AGGATGCTGC TGGCTACCCT GTGGAACACC TACATCTGTA TTAACGAAGC 8760 CTTTCTCAAT GCTCACGCTG TAGGTATCTC AGTTCGGTGT AGGTCGTTCG CTCCAAGCTG 8820 GGCTGTGTGC ACGAACCCCC CGTTCAGCCC GACCGCTGCG CCTTATCCGG TAACTATCGT 8880 CTTGAGTCCA ACCCGGTAAG ACACGACTTA TCGCCACTGG CAGCAGCCAC TGGTAACAGG 8940 ATTAGCAGAG CGAGGTATGT AGGCGGTGCT ACAGAGTTCT TGAAGTGGTG GCCTAACTAC 9000 GGCTACACTA GAAGGACAGT ATTTGGTATC TGCGCTCTGC TGAAGCCAGT TACCTTCGGA 9060 ARAAGAGTTG GTAGCTCTTG ATCCGGCAAA CAAACCACCG CTGGTAGCGG TGGTTTTTTT 9120 GTTTGCAAGC AGCAGATTAC GCGCAGAAAA AAAGGATCTC AAGAAGATCC TTTGATCTTT 9180 TCTACGGGGT CTGACGCTCA GTGGAACGAA AACTCACGTT AAGGGATTTT GGTCATGAGA 9240 9300 TAAAGTATAT ATGAGTAAAC TTGGTCTGAC AGTTACCAAT GCTTAATCAG TGAGGCACCT 9360 ATCTCAGCGA TCTGTCTATT TCGTTCATCC ATAGTTGCCT GACTCCCCGT CGTGTAGATA 9420 ACTACGATAC GGGAGGGCTT ACCATCTGGC CCCAGTGCTG CAATGATACC GCGAGACCCA 9480 CGCTCACCGG CTCCAGATTT ATCAGCAATA AACCAGCCAG CCGGAAGGGC CGAGCGCAGA





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GTCAGAAGTA	AGTTGGCCGC	AGTGTTATCA	CTCATGGTTA	TGGCAGCACT	GCATAATTCT	9840
CTTACTGTCA	TGCCATCCGT	AAGATGCTTT	TCTGTGACTG	GTGAGTACTC	AACCAAGTCA	9900
TTCTGAGAAT	AGTGTATGCG	GCGACCGAGT	TGCTCTTGCC	CGGCGTCAAC	ACGGGATAAT	9960
ACCGCGCCAC	ATAGCAGAAC	TTTAAAAGTG	CTCATCATTG	GAAAACGTTC	TTCGGGGCGA	10020
AAACTCTCAA	GGATCTTACC	GCTGTTGAGA	TCCAGTTCGA	TGTAACCCAC	TCGTGCACCC	10080
AACTGATCTT	CAGCATCTTT	TACTTTCACC	AGCGTTTCTG	GGTGAGCAAA	AACAGGAAGG	10140
CAAAATGCCG	CAAAAAAGGG	AATAAGGGCG	ACACGGAAAT	GTTGAATACT	CATACTCTTC	10200
CTTTTTCAAT	ATTATTGAAG	CATTTATCAG	GGTTATTGTC	TCATGAGCGG	ATACATATTT	10260
GAATGTATTT	AGAAAAATAA	ACAAATAGGG	GTTCCGCGCA	CATTTCCCCG	AAAAGTGCCA	10320
CCTGACGTCT	AAGAAACCAT	TATTATCATG	ACATTAACCT	ATAAAAATAG	GCGTATCACG	10380
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(2) INFORMATION FOR SEQ ID NO:2:

- (i) SEQUENCE CHARACTERISTICS:
 - (A) LENGTH: 20 base pairs
 - (B) TYPE: nucleic acid
 - (C) STRANDEDNESS: single (D) TOPOLOGY: unknown
- (ii) MOLECULE TYPE: other nucleic acid
- (xi) SEQUENCE DESCRIPTION: SEQ ID NO:2:

CATGGTAATA GCGATGACTA

20

(2) INFORMATION FOR SEQ ID NO:3:

- (i) SEQUENCE CHARACTERISTICS:
 - (A) LENGTH: 20 base pairs

 - (B) TYPE: nucleic acid (C) STRANDEDNESS: single (D) TOPOLOGY: unknown
- (ii) MOLECULE TYPE: other nucleic acid
- (xi) SEQUENCE DESCRIPTION: SEQ ID NO:3:

- (2) INFORMATION FOR SEQ ID NO:4:
 - (i) SEQUENCE CHARACTERISTICS:

 (A) LENGTH: 20 base pairs

 (B) TYPE: nucleic acid

 (C) STRANDEDNESS: single

 (D) TOPOLOGY: unknown
 - (ii) MOLECULE TYPE: other nucleic acid
 - (xi) SEQUENCE DESCRIPTION: SEQ ID NO:4:

ATAAGCTGCA ATAAACAAGT